PACT Education Initiative: The Clinical Research Roadmap

The PACT clinical research roadmap has been developed to provide a high-level overview to assist researchers in the identification of the critical areas that need to be considered when bringing a cellular therapy to an Investigational New Drug (IND) application and eventually into the clinic to treat patients.

In brief, the four featured areas discussed in the roadmap are (I) Translational Research, (II) Resource Development, (III) Preclinical Studies, and (IV) IND Filing. The first section of the roadmap highlights the process of translational research in which steps are identified to determine when a therapeutic candidate is ready to enter the translational phase. The second section describes how a study team is coordinated to initiate and plan pre-clinical and clinical research studies aimed at bringing the therapeutic candidate to the clinic. The third section covers developing and implementing translational development and cell product validation to refine and optimize the early preclinical assays and models used during the discovery phase as the therapeutic candidate moves through product lifecycle. Finally, the last section of the roadmap focuses on identifying elements used in developing a regulatory plan during the early stages to facilitate the IND development and submission processes.

The PACT clinical research roadmap is generated from the collaborative knowledge bank and cumulative experiences of the five cell processing facilities of PACT. The key areas outlined above provide guidance to researchers and clinicians to orchestrate initiation, development, and final filing of an IND Application.

The complete clinical roadmap is found on the PACT website: http://www.pactgroup.net/
2013 Product-Related Bibliography: Manuscripts


Lee JW, Krasnodembskaya A, McKenna DH, Song Y, Abbott J and Matthay MA. Therapeutic Effects of Human Mesenchymal Stem Cells In Ex Vivo Human Lungs Injured with Live Bacteria. *Am J Respir Crit Care Med*. 2013 Jan 4 (Epub ahead of print) [PMID: 23292883]


2013 Product-Related Bibliography: Scientific Meeting Abstracts


Process Development for Scalable Manufacturing of HESC-derived Cardiomyocytes. Invited speech at: Biannual Meeting and Symposium of the International Translational and Regenerative Medicine Center; 2013 Jan 17; City of Hope, Duarte, CA.
Successful SOP distribution initiative still strong into 2014

As cellular therapy manufacturing continues to expand in both scope and complexity, PACT has strove to find areas in which to provide quality assistance. Often, there is little support available to establish basic procedures including standard operating procedures (SOPS).

Over the years, PACT has served as a significant resource to the cellular therapy community by providing template SOPs from its high quality manufacturing facilities via request through the PACT website.

Over 125 individual requests for SOPs have been received by PACT since January 2010, with 32 requests having been received in 2013 alone. The requests received this year account for 27% of the total number received.

Overall the most frequent SOP requested in the past year has been quality management followed in order of frequency by validation processes, facility cleaning and environmental monitoring. The country of origin is most frequently the United States followed by Canada, Europe, the UK and the Middle East.

These serve as a reference to the cell therapy community with an ultimate goal to improve the operations and quality of all cell manufacturing. For more information on services available through PACT or to request SOPs visit the PACT website at www.pactgroup.net.

SOPs are currently available in the following categories:

- Cleaning Procedures (Clean)
- Environmental Monitoring (Env)
- Deviation Management (DM)
- Regulatory/Clinical (Reg/C)
- Quality Assurance/Quality Control (QA/QC)
- Personnel Training (Pers)
- Quality Management (Qual)
- Standard Operating Procedures (SOP): Development

PACT Bibliography Updates, continued...

2013 PACT-Related Bibliography: Manuscripts

2013 PACT-Related Bibliography: Scientific Meeting Abstracts
PACT Workshop: “Developing Cellular Therapies: From Preclinical Safety to Clinical Evaluation”

The University of Wisconsin-Madison Waisman Biomanufacturing (WB) hosted a day-long PACT-sponsored workshop, entitled “Developing Cellular Therapies: From Preclinical Safety to Clinical Evaluation” on April 9, 2013. The timing of this event was very carefully planned to complement the 8th Annual Stem Cell Symposium held the following day. The Stem Cell Symposium focused on Cardiac Stem Cell Therapies, and was coordinated by the University of Wisconsin-Madison Stem Cell and Regenerative Medicine Program and the BioPharmaceutical Technology Center Institute.

WB was the 4th PACT facility to host the PACT-sponsored workshop which focused on providing information to aid investigators in moving their basic research through the translational phase and into clinical trials. This conference featured a range of topics from translational and clinical research to more basic instruction on technical cell product development. The workshop highlighted cellular product development in the areas of heart, lung, and blood. Speakers included Dr. Cliona Rooney (Baylor College of Medicine), Dr. Sean Savitz (University of Texas Medical School at Houston), Dr. Michael Matthay (University of California San Francisco School of Medicine), Dr. Christian Capitini (University of Wisconsin School of Medicine & Public Health), and Dr. Timothy Kamp (University of Wisconsin School of Medicine & Public Health).

The workshop included a panel of speakers focusing on the transition to clinical development, touching on the challenges for animal models (Dr. Timothy Hacker, UW-Madison), cell delivery approaches (Dr. Marlowe Eldridge, UW-Madison), product characterization (Dr. Peiman Hematti, UW-Madison), product manufacture and technology transfer (Dr. Derek Hei, Waisman Biomanufacturing, UW-Madison), as well as FDA communications and IND filings (John Centanni, M.S., UW-Madison). The workshop garnered an enthusiastic group of approximately 90 attendees, including participants from the east and west coasts. Those attending the workshop commented on both the benefit of the topics presented, and the speakers’ in-depth knowledge on their particular area of research and expertise. The University of Wisconsin-Madison PACT team was energized by this response and plans to organize future events focused on the development of novel therapeutics, and the challenges that need to be addressed in order to advance these into the clinic.

PACT Cell Processing Facilities:
- Baylor College of Medicine Center for Cell and Gene Therapy
  Contract Number: HHSN268201000007C
- University of Minnesota Molecular and Cellular Therapeutics Facility
  Contract Number: HHSN268201000008C
- Center for Human Cell Therapy Boston
  Contract Number: HHSN268201000009C
- University of Wisconsin - Madison Waisman Biomanufacturing Facility
  Contract Number: HHSN268201000010C
- City of Hope Center for Applied Technology Development
  Contract Number: HHSN268201000011C

PACT Coordinating Center:
- The EMMES Corporation
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